WORLD INTELLECTUAL PROPERTY ORGANIZATION International Bureau



(51) International Patent Classification ⁶ : C12N 15/86, 5/10, A61K 48/00, A01K 67/027		(11) International Publication Number: WO 97/09441		
		(43) International Publication Date: 13 March 1997 (13.03.97		
21) International Application Number: PCT/US	(74) Agent: DUGAN, Deborah, A., Genzyme Corporation, One Mountain Road, Framingham, MA 01701 (US).			
22) International Filing Date: 6 September 1996 (06.09.9	/ 		
30) Priority Data: 60/003,470 8 September 1995 (08.09.95	(81) Designated States: AU, CA, JP, US, European patent (AT, BE CH, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL PT, SE).			
60) Parent Application or Grant (63) Related by Continuation US Filed on 8 September 1995 (6	Published Without international search report and to be republished upon receipt of that report.			
(71) Applicant (for all designated States except US): GE CORPORATION [US/US]; One Mountain Road, ham, MA 01701 (US).	ENZYM Framin			
(72) Inventors; and (75) Inventors/Applicants (for US only): WADSWORTH C. [US/US]; 10 Straw Hollow Lane, Shrewsb 01545 (US). VINCENT, Karen [US/US]; 337 Park Arlington, MA 02174 (US). PIRAINO, Susan Apartment 411B, 1622 Worcester Road, Framingl 01701 (US). KYOSTIO, Sirkka [FI/US]; 61 Wint Framingham, MA 01701 (US).	oury, M k Avent (US/US ham, M			

(57) Abstract

The present invention is directed to methods for generating high titer, contaminant free, recombinant AAV vectors, methods and genetic constructs for producing AAV recombinant vectors conveniently and in large quantities, methods for the delivery of all essential viral proteins required in trans for high yields of recombinant AAV, recombinant AAV vectors for use in gene therapy, novel packaging cell lines which obviate the need for cotransfection of vector and helper plasmids, helper plasmids and vector plasmid backbone constructs, a reporter assay for determining AAV vector yield. Further provided are recombinant AAV vectors in a pharmaceutically acceptable carrier, methods of delivering a transgene of interest to a cell, compositions and methods for delivering a DNA sequence encoding a desired polypeptide to a cell, and transgenic non-human mammals that express a human chromosome 19 AAV integration locus.

FOR THE PURPOSES OF INFORMATION ONLY

Codes used to identify States party to the PCT on the front pages of pamphlets publishing international applications under the PCT.

AM	Armenia	GB	United Kingdom	MW	Malawi
AT	Austria	GE	Georgia	MX	Mexico
AU	Australia	GN	Guinea	NE	Niger
BB	Barbados	GR	Greece	NL	Netherlands
BE	Belgium	HU	Hungary	NO	Norway
BF	Burkina Faso	1E	Ireland	NZ	New Zealand
BG	Bulgaria	IT	Italy	PL	Poland
BJ	Benin	JP	Japan	PT	Portugal
BR	Brazil	KE	Kenya	RO	Romania
BY	Belarus	KG	Kyrgystan	RU	Russian Federation
CA	Canada	KP	Democratic People's Republic	SD	Sudan
CF	Central African Republic		of Korea	SE	Sweden
CG	Congo	KR	Republic of Korea	SG	Singapore
CH	Switzerland	KZ	Kazakhstan	SI	Slovenia
CI	Côte d'Ivoire	LI	Liechtenstein	SK	Slovakia
CM	Cameroon	LK	Sri Lanka	SN	Senegal
CN	China	LR	Liberia	SZ	Swaziland
cs	Czechoslovakia	LT	Lithuania	TD	Chad ·
CZ	Czech Republic	LU	Luxembourg	TG	Togo
DE	Germany	LV	Latvia	TJ	Tajikistan
DK	Denmark	MC	Monaco	TT	Trinidad and Tobago
EE	Estonia	MD	Republic of Moldova	UA	Ukraine
ES	Spain	MG	Madagascar	UG	Uganda
FI	Finland	ML	Mali	US	United States of America
FR	France	MN	Mongolia	UZ	Uzbekistan
GA	Gabon	MR	Mauritania	VN	Viet Nam